

Abstract (lay version) of project

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Identifying targets for therapy in a novel genetic *Xenopus* model for desmoid tumor formation

This project aims to provide a fast, semi-high throughput and cheap animal model for identifying and/or characterizing promising drug targets for treating desmoid tumors. In addition the platform allows pre-clinical assessment of novel candidate therapeutic compounds. The project builds on the recent introduction of efficient genome editing methods using TALEN and CRISPR/Cas9, which are creating unique and unmatched opportunities in several research fields, including cancer research. For the first time it is now possible to create functional gene knockouts in a number of model organisms. We have recently generated the first genetic tumor model in the organism *Xenopus tropicalis*. Because of the external development of the *Xenopus* embryo and its diploid genome, gene targeting experiments using CRISPR/Cas9 or TALEN are extremely efficient and cheap. Interestingly, when locally targeting the tumor suppressor gene APC we generated tadpoles that rapidly (< 1.5 months) and efficiently (>90%) developed desmoid tumors. This model presents a unique and novel experimental platform that (i) allows the rapid screening and evaluation of genes that contribute to the growth of the tumor, (ii) could serve to assess the clinical relevance of novel drug targets for treating desmoid tumors and (iii) can be used as a preclinical drug screening/assessment. We believe that our model offers a unique experimental platform that can be easily plugged into the research lines of several groups active in the field.